4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0386]

Agency Information Collection Activities; Proposed Collection; Comment Request; Orphan Drugs

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing an opportunity for public comment on the proposed collection of certain information by the Agency. Under the Paperwork Reduction Act of 1995 (PRA), Federal Agencies are required to publish notice in the *Federal Register* concerning each proposed collection of information, including each proposed extension of an existing collection of information, and to allow 60 days for public comment in response to the notice. This notice solicits comments on information collection associated with Orphan Drug provisions of the Federal Food, Drug, and Cosmetic Act (FD&C Act) and implementing regulations.

DATES: Submit either electronic or written comments on the collection of information by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*].

ADDRESSES: You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*]. The https://www.regulations.gov electronic filing system will accept comments

until 11:59 p.m. Eastern Time at the end of [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*]. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: https://www.regulations.gov. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to https://www.regulations.gov will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on https://www.regulations.gov.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2014-N-0386 for "Agency Information Collection Activities; Proposed Collection; Comment Request; Orphan Drugs." Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at https://www.regulations.gov or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday, 240-402-7500.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on https://www.regulations.gov. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as

"confidential." Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at: https://www.govinfo.gov/content/pkg/FR-2015-09-18/pdf/2015-23389.pdf.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to https://www.regulations.gov and insert the docket number, found in brackets in the heading of this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852, 240-402-7500.

FOR FURTHER INFORMATION CONTACT: Jonna Capezzuto, Office of Operations, Food and Drug Administration, Three White Flint North, 10A-12M, 11601 Landsdown St., North Bethesda, MD 20852, 301-796-3794, PRAStaff@fda.hhs.gov.

SUPPLEMENTARY INFORMATION: Under the PRA (44 U.S.C. 3501-3521), Federal Agencies must obtain approval from the Office of Management and Budget (OMB) for each collection of information they conduct or sponsor. "Collection of information" is defined in 44 U.S.C. 3502(3) and 5 CFR 1320.3(c) and includes Agency requests or requirements that members of the public submit reports, keep records, or provide information to a third party. Section 3506(c)(2)(A) of the PRA (44 U.S.C. 3506(c)(2)(A)) requires Federal Agencies to provide a 60-day notice in the *Federal Register* concerning each proposed collection of information, including each proposed extension of an existing collection of information, before submitting the collection to OMB for approval. To comply with this requirement, FDA is publishing notice of the proposed collection of information set forth in this document.

With respect to the following collection of information, FDA invites comments on these topics: (1) whether the proposed collection of information is necessary for the proper performance of FDA's functions, including whether the information will have practical utility; (2) the accuracy of FDA's estimate of the burden of the proposed collection of information, including the validity of the methodology and assumptions used; (3) ways to enhance the quality, utility, and clarity of the information to be collected; and (4) ways to minimize the burden of the collection of information on respondents, including through the use of automated collection techniques, when appropriate, and other forms of information technology.

Orphan Drugs; 21 CFR Part 316

OMB Control Number 0910-0167--Revision

This information collection supports FDA regulations implementing sections 525, 526, 527, and 528 of the FD&C Act, as well as related guidance. Sections 525, 526, 527, and 528 pertain to the development of drugs for rare diseases or conditions, including biological products and antibiotics, otherwise known or referred to as "Orphan Drugs." Specifically, section 525 of the FD&C Act (21 U.S.C. 360aa) requires written recommendations on studies required for approval of a marketing application for a drug for a rare disease or condition. The information collection in 21 CFR 316.10, 316.12, and 316.14 is approved under OMB control numbers 0910-0001 and 0910-0014. Section 526 of the FD&C Act (21 U.S.C. 360bb) provides for designation of drugs as orphan drugs when certain conditions are met; section 527 (21 U.S.C. 360cc) provides conditions under which a sponsor of an approved orphan drug enjoys exclusive FDA marketing approval for that drug for the orphan indication for a period of 7 years; and finally, section 528 (21 U.S.C. 360dd) is intended to encourage sponsors to make investigational orphan drugs available for treatment of persons in need on an open protocol basis before the drug has

been approved for general marketing. Open protocols may permit patients who are not part of the formal clinical investigation to obtain treatment where adequate supplies exist and no alternative effective therapy is available.

We have issued regulations in part 316 (21 CFR part 316) to implement the Orphan Drug provisions of the FD&C Act and to set forth procedures and requirements related to requesting recommendations for investigations of drugs for rare diseases or conditions; requesting designation of a drug for a rare disease or condition; or requesting exclusive approval for a drug for a rare disease or condition. To assist respondents and consistent with 21 CFR part 316.50, our Office of Orphan Products Development (OOPD) maintains and makes publicly available guidance documents that apply to the Orphan Drug provisions of the FD&C Act and regulations in part 316. The list is maintained on the internet and guidance documents are issued in accordance with our Good Guidance Practice regulations in 21 CFR part 10.115 which provide for public comment at any time. Accordingly, we are revising the information collection to include Agency guidance. The document entitled, "Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff on Meetings With the Office of Orphan Products Development," provides recommendations to industry, researchers, patient groups, and other stakeholders interested in requesting a meeting, including a teleconference, with OOPD on issues related to orphan drug designation requests, humanitarian use device designation requests, rare pediatric disease designation requests, funding opportunities through the Orphan Products Grants Program and the Pediatric Device Consortia Grants Program, and orphan product patient related topics of concern. It is also intended to assist OOPD staff in addressing such meeting requests. This guidance describes procedures for requesting, preparing, scheduling, conducting, and documenting such meetings, and discusses background information we recommend be

included in such requests. Information collection attendant to recommendations in the guidance are currently approved under OMB control number 0910-0787, however for efficiency of Agency operations we are consolidating it into this related information collection. The guidance is available at https://www.fda.gov/media/92815/download.

The FDA Orphan Drug Designation Request Form (Form FDA 4035) is intended to benefit sponsors who desire to seek orphan designation of drugs intended for rare diseases or conditions from only FDA. The form is a simplified method for sponsors to provide only information required by 21 CFR 316.20 for FDA to make a decision.

During this public health emergency associated with the COVID-19 pandemic, the OOPD is providing sponsors with increased flexibility for submission of orphan drug designation requests and related submissions (amendments, annual reports, etc.). During this public health emergency, orphan drug designation, humanitarian use device designation, and rare pediatric disease designation requests and submissions may be submitted electronically by email to the OOPD. When transmitting information to the Orphan Drug Designation Program via email, please utilize the mailbox orphan@fda.hhs.gov. The use of automated read receipt is recommended to avoid the need to call to verify receipt of the email. Sponsors and others who plan to email information to FDA that is considered to be private, sensitive, proprietary, or commercial confidential are strongly encouraged to send it from an FDA secured email address so the transmission is encrypted. The OOPD will assume that the addresses of emails received or email addresses provided as a point of contact are FDA secure when responding to those email addresses.

We estimate the burden of the information collection as follows:

Table 1.--Estimated Annual Reporting Burden[1]

21 CFR Section	No. of Respondents	Annual Frequency per Response	Total Annual Responses	Hours per Response	Total Hours
Content and format of a request for designation; request for verification of status; amendment to designation	534	1.25	668	135	90,180
§§ 316.20, 316.21, 316.26 (Form FDA 4035)	534	1.25	668	32	21,376
§ 316.22; Notifications of changes in agents	132	1	70	2	264
§ 316.24(a); Deficiency letters and granting orphan-drug designation	20	1	20	2	40
§ 316.27; Submissions to change ownership of orphan-drug designation	104	1	104	5	520
§ 316.30; Annual reports	744	1	744	3	2,232
§ 316.36; Assurance of the availability of sufficient quantities of the orphan drug; holder's consent for the approval of other marketing applications for the same drug	1	3	3	15	45
Guidance Recommendations: Meeting requests to OOPD and related submission packages	2,508	1	2,508	3.595	9,016
Total					123,623

[1] There are no capital costs or operating and maintenance costs associated with this collection of information.

Based on our evaluation, we have adjusted the currently approved burden estimate we attribute to information collection activities associated with our Orphan Drug program to reflect an increase in submissions.

Dated: September 28, 2020.

Lauren K. Roth,

Associate Commissioner for Policy.

[FR Doc. 2020-21843 Filed: 10/1/2020 8:45 am; Publication Date: 10/2/2020]